Bio
BIOTECHNOLOGY INDUSTRY ORGANIZATION

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Jane E. Henney, MD
Commissioner,
Food and Drug Administration
5600 Fishers Lane
Rockville, MD 20857

Dear Dr. Henney:

FDA and pediatricians have expressed concern that there is insufficient information on drug labels regarding pediatric use. Section 111 of the Food and Drug Modernization Act of 1997 (FDAMA) established one solution to this problem, an incentive program to encourage pediatric studies on new and marketed drugs. On December 2, 1998, FDA issued regulations requiring manufacturers to assess the safety and effectiveness of new drugs and biological products in pediatric populations (63 Federal Register 66632). This rule represents a different solution to the problem; indeed, a mandatory solution that, as stated in the rule's preamble, FDA believes is necessary because FDAMA may not ensure that all necessary pediatric studies will be carried out.

BIO supports the goal of providing adequate pediatric information in drug labels for those products having a sufficient pediatric population. This rule raises some serious concerns, however. FDA's legal authority to mandate specific clinical trials in specific populations or sub-populations is questionable, as is the wisdom of the rule from a broader policy standpoint. Ultimately, the value of the rule will be judged on the basis of how FDA chooses to implement it. In the interest of cooperation, therefore, we choose not to dwell on the legal ambiguity at this time and, instead, direct our comments to implementation policy.

How FDA implements the rule may have important consequences not only for pediatric patients but also for the biotechnology industry and our member companies. We believe that the interests of pediatric patients will be harmed in the long run by implementation policies that impact the biotechnology industry adversely. Ill-advised application of the rule could stifle innovation and lead to results quite at odds with the rule's intent. Because the rule is mandatory, we urge FDA to be especially circumspect and judicious in applying it.

First, FDA should steer clear of any implementation policy that could have the effect of ceding to the government control over drug development. Government

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direction of private resources is at odds with the market-based system that has produced the great advances in biomedical research that the American people benefit from today. Decisions on how scarce private research resources are spent should remain, we respectfully submit, the purview of the private sector.

Second, application of the rule should be limited to situations in which the need for additional pediatric information is demonstrable by actual clinical data, rather than simply deemed to be subjectively desirable. Otherwise, the important distinction between safety and manufacturer's evaluation of markets and indications will be lost, and the government will have commandeered decisions concerning which drugs to develop for which markets. Such a policy would create an extremely inhospitable environment for our member companies to conduct research, raise venture capital, and attract talented scientists.

Third, for many products, it is unclear whether there is a sufficient pediatric population to warrant development. For other products, selection of the appropriate patient population and the number of pediatric patients to be studied should be determined in light of the anticipated benefits to be derived from the studies. FDA must recognize that no amount of labeling will ever cover all drugs in all population sub-groups and that further clinical study has a point of diminishing returns.

Fourth, while there may be cases in which the safety profile of a potential new drug is well established and a simple pharmacokinetic study is sufficient to support pediatric labeling, this will not always be the case. We are concerned that rigid application of the rule might delay new product approvals for the broader population, particularly if new formulations or distinct pediatric clinical protocols must be developed. We believe that a better approach is to make liberal use of the deferral provisions of the rule to permit sponsors to carry out pediatric studies following licensure. Smaller biotechnology companies would derive particular benefit from such a policy, since they may not have the resources to conduct pediatric studies before licensure.

Fifth, BIO urges FDA to engage in constructive communication with stakeholders concerning the effects of the rule and the consequences of its implementation policies. We offer our cooperation and our commitment to be a full participant in any dialogue that FDA chooses to conduct.

We respectfully urge restraint, balance, and judicious policy-making on FDA, because we believe that the long-term interests of the nation's children will be served most effectively by a commercial and regulatory environment in which the biotechnology industry can continue to develop innovative therapies and cures.

Sincerely,

Carl B. Feldbaum
President